

Unblocking the bottleneck – better trial design in progressive multiple sclerosis.

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A major issue in trying to alter the gradient of progression in multiple sclerosis (MS) is the time and expense of phase 2 programmes, both investigator and pharma-led. There is no shortage of promising molecules and/or pathways to be explored, but each trial is likely to take at least 5 years from set-up to final result. Moreover, without a well conducted phase 2 trial, the chance at success at phase 3 is correspondingly reduced. Quicker, more efficient and different trial design structures must be found and implemented to reduce the timeline, both for drug development and non-drug intervention (eg physiotherapy).

Here I will explore some of those methods including: multi-arm, adaptive, n of 1, Simon-2-stage, and Bayesian concepts. Examples will be given, looking at previous experience, as well as what could and should be done in the future.